December 30, 2016

OPKO Health Provides Update on hGH-CTP Clinical Programs

- Commenced data analysis of the Phase 3 Clinical Study of hGH-CTP in Growth Hormone Deficient Adults

- Initiated Global Pediatric Phase 3 Clinical Study of hGH-CTP in Growth Hormone Deficient Children

MIAMI, Dec. 30, 2016 (GLOBE NEWSWIRE) -- OPKO Health, Inc. (NASDAQ:OPK) announced it has commenced data analysis in the phase 3, double-blind, placebo-controlled study of its investigational long-acting human growth hormone product (hGH-CTP) in adults with growth hormone deficiency (GHD). The multinational, multi-center study, which utilized a 2:1 randomization between hGH-CTP and placebo, enrolled 203 subjects, 198 of whom received at least one dose of study treatment. Treatment was administered through a weekly injection.

On the primary endpoint of change in trunk fat mass from baseline to 26 weeks, there was no statistical difference between hGH-CTP and placebo. However, after unblinding the study, OPKO identified one or more outliers that may have affected the primary outcome. As a result, OPKO is undertaking further review of the study population as promptly as possible. The safety profile observed in this study was consistent with that known for growth hormone treatments. A greater percentage of subjects on hGH-CTP normalized serum concentrations of insulin-like growth factor-I compared to placebo. Additional efficacy and safety data and analyses from the study will be released once available.

In addition to completing the Phase 3 adult GHD study, OPKO announces that it has initiated a pivotal Phase 3 study this month in pre-pubertal growth hormone deficient children to evaluate weekly treatment with hGH-CTP versus daily injections of Genotropin. The hGH-CTP will be delivered in a pen device in this multi-regional study.

OPKO has a world-wide collaboration and license agreement with Pfizer Inc. for the development and commercialization of hGH-CTP. Under the agreement, OPKO is responsible for conducting the clinical program and Pfizer is responsible for registering and commercializing the product.

About OPKO Health, Inc.

OPKO Health is a diversified healthcare company that seeks to establish industry-leading positions in large, rapidly growing markets. Our diagnostics business includes Bio-Reference Laboratories, the nation's third-largest clinical laboratory with a core genetic testing business and a 420-person sales force to drive growth and leverage new products, including the 4Kscore® prostate cancer test and the Claros® 1 in-office immunoassay platform. Our pharmaceutical business features RAYALDEE, an FDA-approved treatment for SHPT in stage 3-4 CKD patients with vitamin D insufficiency, VARUBI™ for chemotherapy-induced

nausea and vomiting (oral formulation launched by partner TESARO and IV formulation PDUFA date: January 2017), TT401, a once or twice weekly oxyntomodulin for type 2 diabetes and obesity which is a clinically advanced drug candidate among the new class of GLP-1 glucagon receptor dual agonists, and TT701, an androgen receptor modulator for androgen deficiency indications. Our biologics business includes hGH-CTP, a once-weekly human growth hormone injection (in phase 3 and partnered with Pfizer), a long-acting Factor VIIa drug for hemophilia (in phase 2a) and a long-acting oxyntomodulin for diabetes and obesity (in phase 1). We also have production and distribution assets worldwide, multiple strategic investments and an active business development strategy. More information is available at www.opko.com.

This press release contains "forward-looking statements," as that term is defined under the Private Securities Litigation Reform Act of 1995 (PSLRA), which statements may be identified by words such as "expects," "plans," "projects," "will," "may," "anticipates," "believes," "should," "intends," "estimates," and other words of similar meaning, including statements regarding timing for completion of data analysis for the study and release of additional safety and efficacy data, whether any outlier(s) impacted the primary outcome and secondary endpoints, whether OPKO's clinical trials for hGH-CTP in adult and pediatric GHD patients will be successful or generate data to support marketing approval, whether study results will demonstrate hGH-CTP is superior to placebo in reducing truncal fat, whether hGH-CTP will prove to be safe and effective and achieve IGF-1 normalization, whether hGH-CTP will be successfully developed or commercialized, expectations regarding the product and its market potential, as well as other non-historical statements about our expectations, beliefs or intentions regarding our business, technologies and products, financial condition, strategies or prospects. Many factors could cause our actual activities or results to differ materially from the activities and results anticipated in forward-looking statements. These factors include those described in our filings with the Securities and Exchange Commission, as well as the risks inherent in funding, developing and obtaining regulatory approvals of new, commercially-viable and competitive products and treatments. In addition, forward-looking statements may also be adversely affected by general market factors, competitive product development, product availability, federal and state regulations and legislation, the regulatory process for new products and indications, manufacturing issues that may arise, patent positions, litigation, and the success of our collaboration on hGH-CTP with Pfizer, Inc. among other factors. The forward-looking statements contained in this press release speak only as of the date the statements were made, and we do not undertake any obligation to update forward-looking statements. We intend that all forward-looking statements be subject to the safe-harbor provisions of the PSLRA.

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